From bench to bedside through industry collaborations

Annamaria Merico, Head of Technology Transfer Telethon Foundation

FONDAZIONE



Telethon Mission & Vision

- Fondazione Telethon is a major Italian biomedical charity focused on genetic diseases
- Founded in 1990 at the behest of a group of patients
- Supported through fundraising

OUR MISSION

Advance biomedical research towards the cure of genetic diseases

592,5 M€ research investment14.212 papers published

1.640 PIs awarded**584** genetic diseases studied

OUR VISION

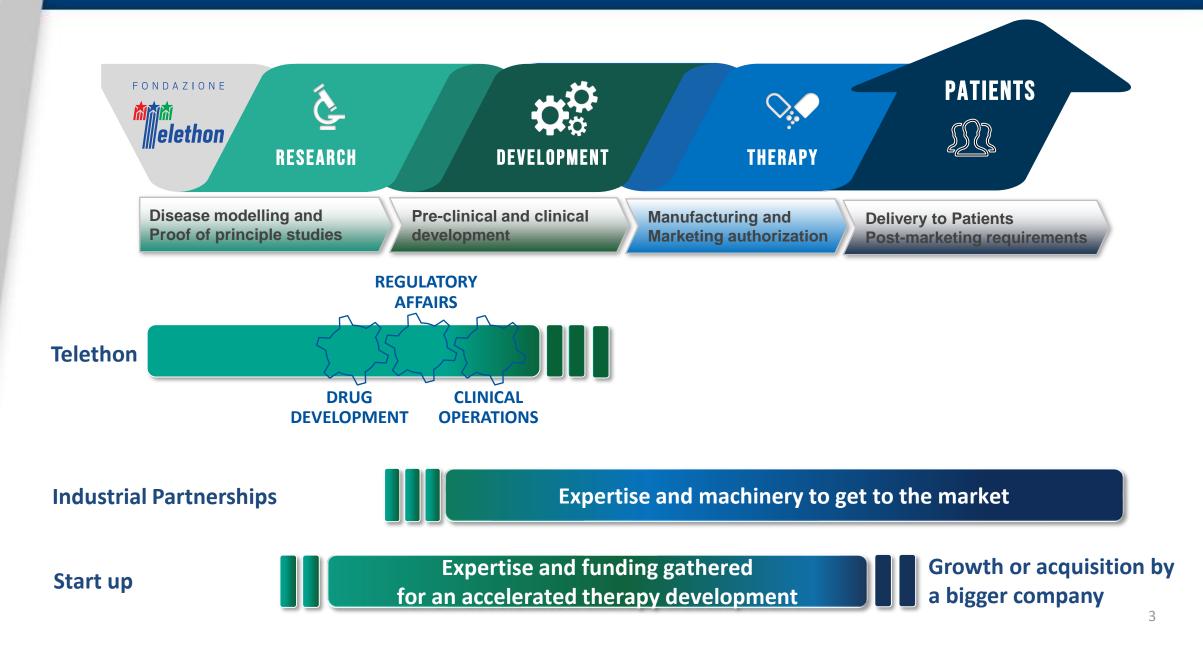
Convert the results of excellent, selected and sustained research into available therapies

130 patients treated

2 Therapies approved



Teaming up with industry and investors



Telethon supports derisking activities to accelerate development

R&D

Regulatory Affairs and Clinical Operation

Clinical

Trials

GCP

GMP

Pre-clinical

development

GLP



Creation of **Process Development Laboratory** to develop protocols for the production of new gene and cell therapy products, ensuring a smoother technology transfer to GMP facilities

Creation of **GLP-certified laboratories** for the conduct of **pre-clinical studies** compliant with regulatory requirements (quality and integrity of data)

Creation of a **Clinical Trial Office**, with **dedicated personnel** in support of start, management and closure of ATMP clinical trials and **training** to specialised staff (e.g. clinical nurses, data manager, etc.)

Identification of a GMP-certified **Contract Manufacturing Organisation**; high costs and timing; risk of failure

therapies

Market &

Access

(Access)

"Just like home": a

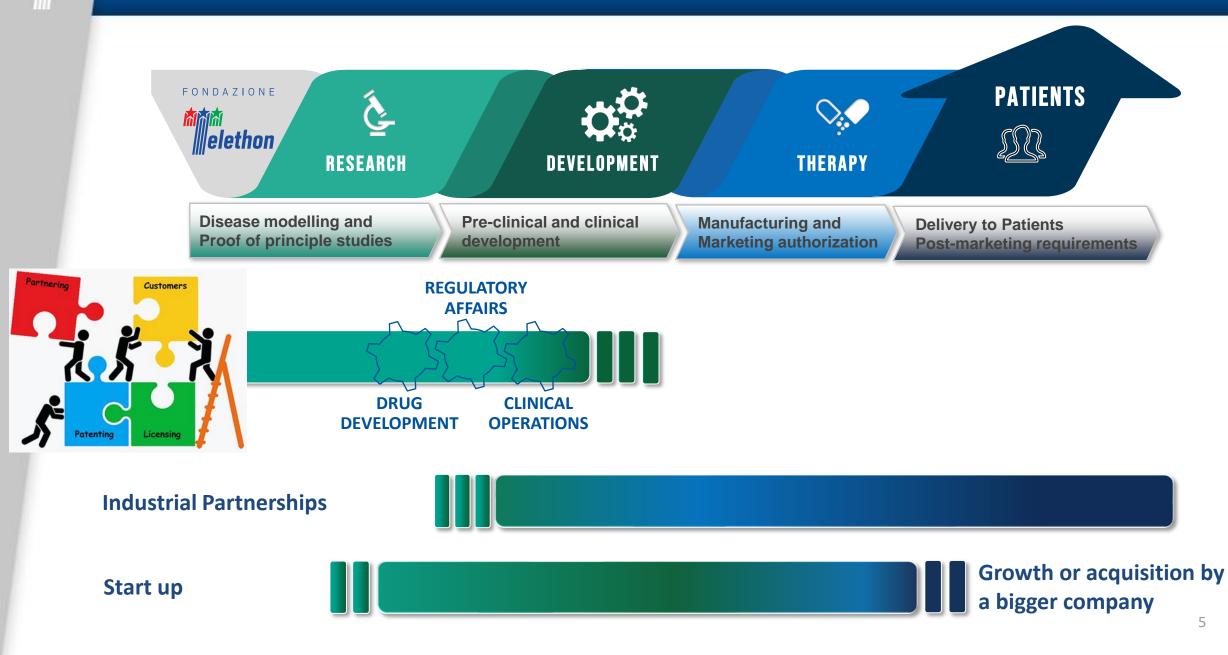
multidisciplinary team to assist

families participating in gene

None of these actions are typical of a charity



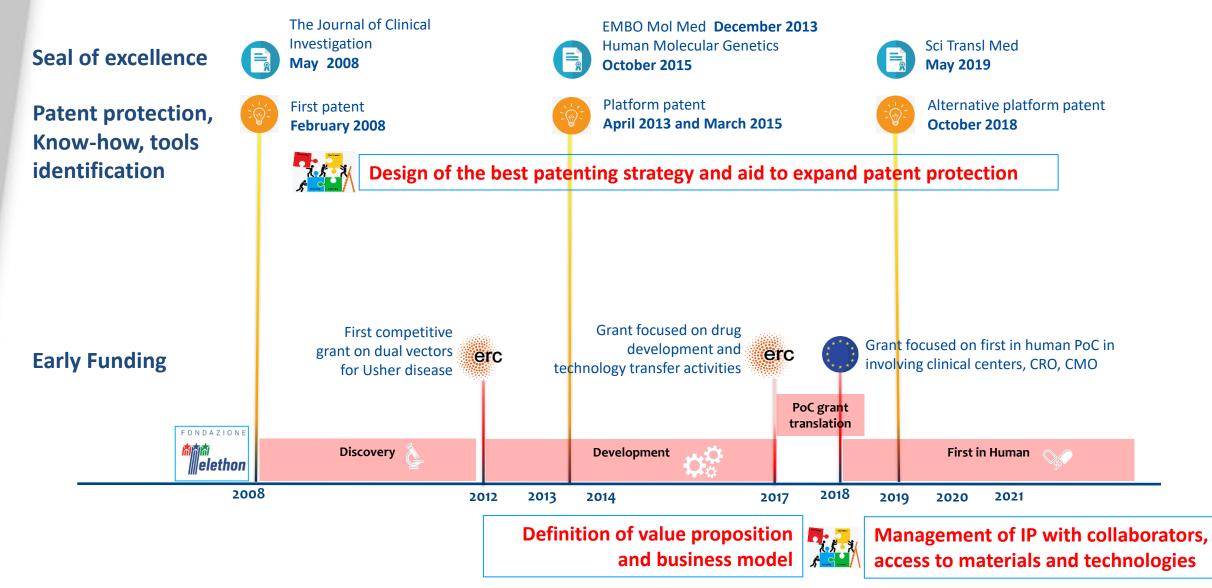
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A long journey guided by industry development vision

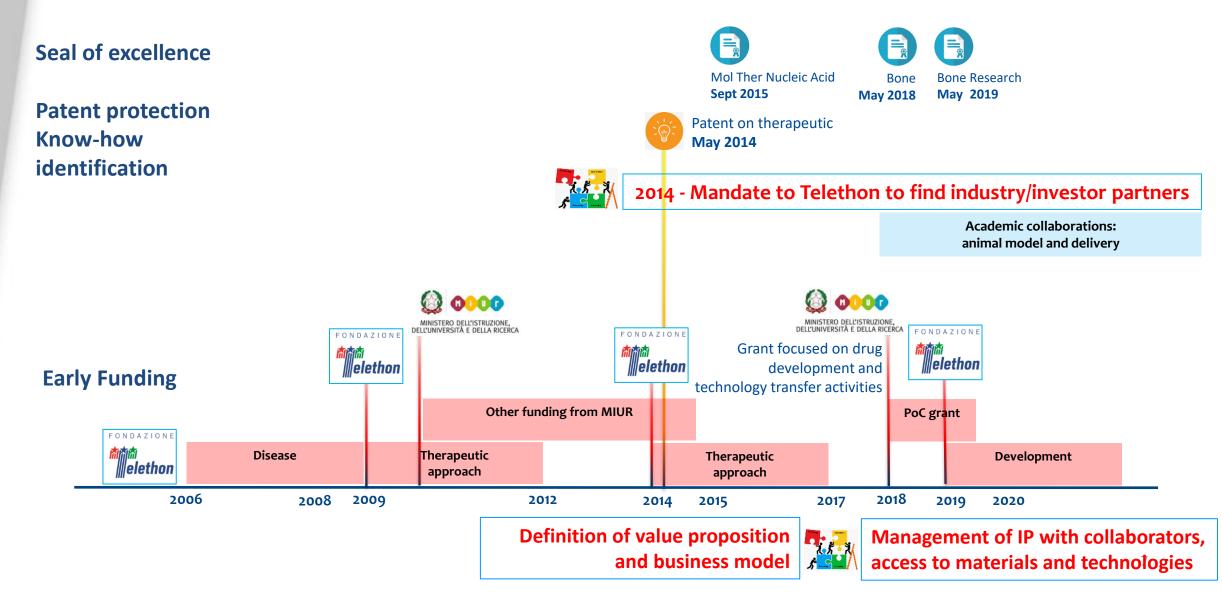


Therapy for rare eye diseases



The partnership between Scientist and TTO is critical

Therapy for rare bone diseases



Why early management of IP is relevant?

It relates to research results (data and materials):

- 1. Is your Institution the owner of the results?
- 2. Can you freely use the results?
- 3. Can you freely publish the results?
- 4. Can you use the results also in collaboration with others?
- 5. Can your Institution exploit the results in industrial partnerships and startup?

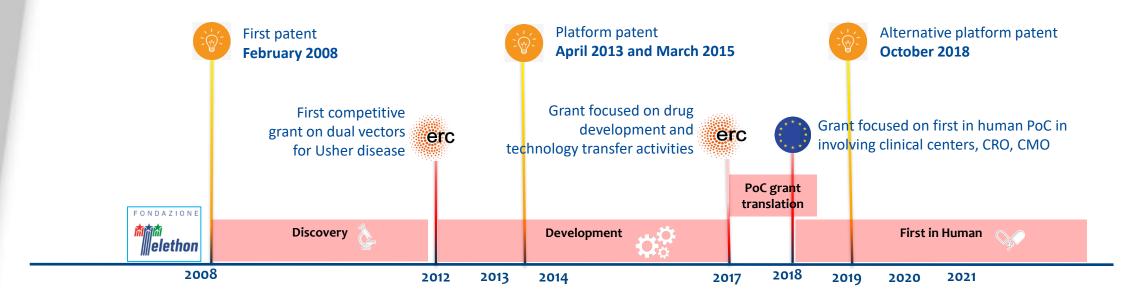
TTO tries to get this degree of freedom!



Partnerships always trigger a learning process



Therapy for rare eye diseases





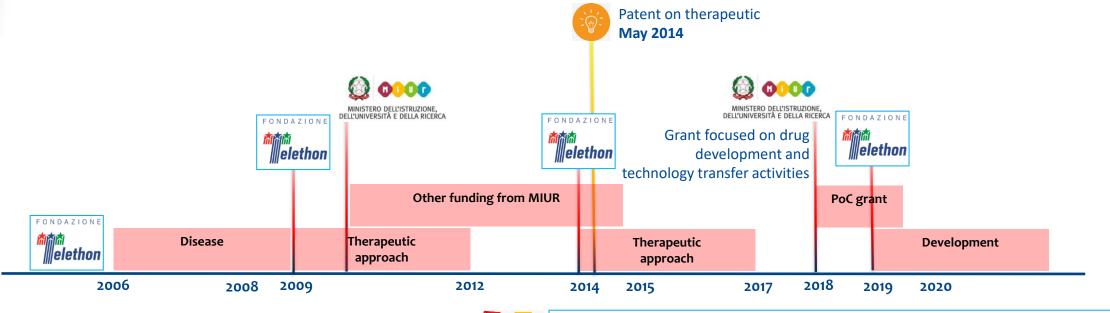
Biotech Company: Research collaboration, option and license agreement Signed in July 2014, option exercised Nov 2016



BIO

Technology transfer is not a one-shot activity







2014 - Mandate to Telethon to find industry/investor partners

13 diligence assessments: main gaps are delivery and drug optimization

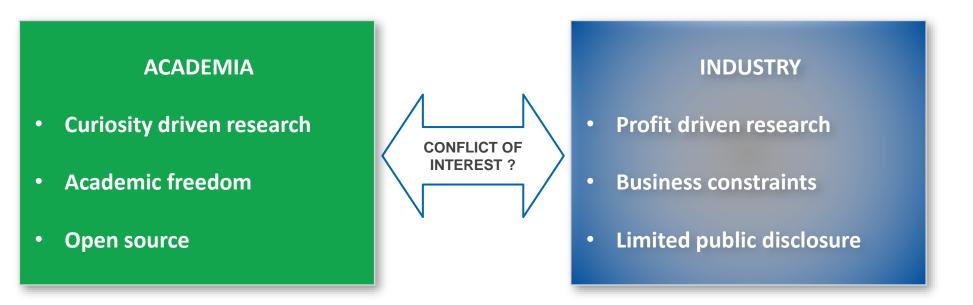
mRNA Company: Material Transfer and Evaluation Agreement Signed Feb 2018, extended to 2023

> Delivery Company: Research collaboration, option and license agreement Signed July 2020, option exercised September 2021

Ongoing efforts to merge the companies competences towards the best drug

Is it possible to partner with industry?

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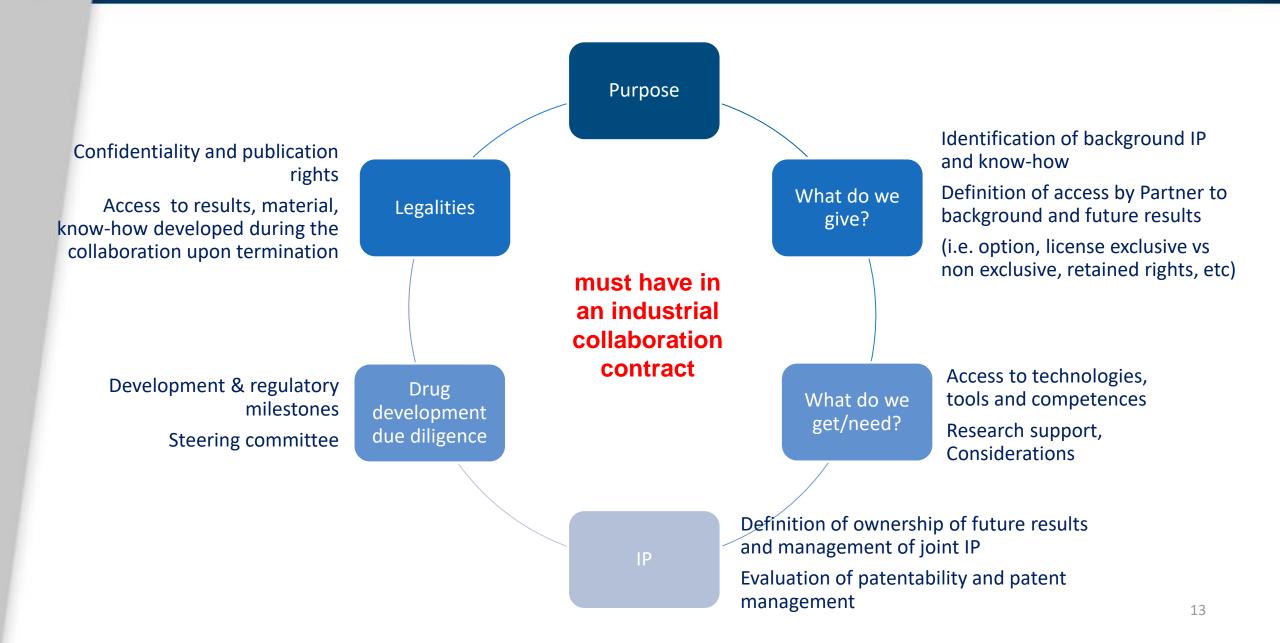
The pillars of technology transfer

TTO should work **in strict cooperation with scientists**, acknowledging their crucial roles in research development

- Safeguard research independence
- Retain intellectual property rights to safeguard opportunities of development
- In case the partner fails to pursue therapy development, revert both IP and co-developed results back to FT
- Partnerships should provide adequate research funding support
- **Partnerships should** supply **additional funding** through development and commercial milestones and royalties



Scientist can contribute to negotiation

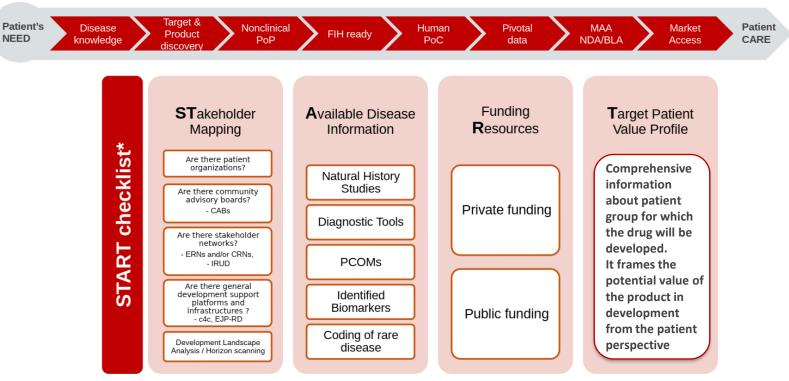


IRDIRC Orphan Drug Development Guidebook: now available interactive!



A simple patient-focused guidebook that <u>describes the available tools, incentives,</u> <u>resources and practices</u> specific for developing traditional and innovative drugs/therapies for rare disease indications and <u>how to best use them</u>.

How to start your orphan drug development



* If this information not available, the first step is to build up the stakeholder mapping, and then generate data to the best of the possibilities

THANKS!

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